CIRM Funded Clinical Trials

A Phase 1/2 Study to Assess the Safety, Tolerability, and Efficacy of ST-400 Autologous HSPC Transplant in Transfusion-dependent β-Thalassemia

Disease Area:	Beta Thalassemia
Investigator:	Ed Conner
Institution:	Sangamo BioSciences, Inc.
CIRM Grant:	CLIN2-11031 (Pre-Active)
Award Value:	$8,000,000
Trial Sponsor:	Sangamo BioSciences, Inc.
Trial Stage:	Phase 1/2
Trial Status:	Launching
Targeted Enrollment:	N/A

Ed Conner

Details:
Sangamo, Inc. is testing genetically engineered blood stem cells for the treatment of beta-thalassemia, a severe form of anemia caused by mutations in the hemoglobin gene. This genetic disorder requires life-long blood transfusions and carries a life expectancy of only 30-50 years. The Sangamo therapy takes a patient's own blood stem cells and, using a gene-editing technology called zinc finger nuclease (ZFN), provides a functional copy of the hemoglobin gene. These modified cells are given back to the patient which potentially will eliminate the need for chronic transfusions and its associated complications.

Design:
Open label, single arm study

Goal:
Safety and tolerability. Efficacy, change from baseline HbF levels, frequency and volume of RBC transfusions.

News Releases:
Sangamo Announces $8 Million CIRM Grant For ST-400 -- A Gene-Edited Cell Therapy Candidate -- To Treat Transfusion-Dependent Bet

Source URL: https://www.cirm.ca.gov/clinical-trial/phase-12-study-assess-safety-tolerability-and-efficacy-st-400-autologous-hspc